IPFA Perspective

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IPFA:
International Plasma Fractionation Association

- **Not-for-Profit Organizations**
- **Operate in commercial, competitive environments**
- **Generally small-medium enterprises**
- **Primarily national suppliers of plasma products**
- **Include niche, low volume, orphan drugs**

IPFA:
International Plasma Fractionation Association

- **Compliance with regulatory requirements**
- **For ‘economic health’ have had to become more international**
- **Same CMC and clinical requirements**
- **Several in Europe have some experience with FDA requirements**
- **Not all could participate in this meeting**

Rare Plasma Protein Disorders and Coagulation Products in Europe

- **Some IPFA members have national products**
  - **FVII** **2 members** **1 country each**
  - **FXI** **2 members** **1 country each**
  - **Protein C** **1 member** **1 country**
  - **AT** **3 members** **1 country each**

Rare Plasma Protein Disorders and IgG Products in Europe

- **Some IPFA members have national products**

– CMV	1 member	1 country
– Rabies	1 member	1 country
– Rubella	2 members	1 country each
– HepB i.m.	2 members	1 country each
– VZV	3 members	1 country each
– HepB i.v.	4 members	1 country each
– Tetanus	4 members	1 country each

Rare Plasma Protein Disorders

- Some IPFA members have national products
- National markets too small for cost-effective production
- Limitations for international licensure
  - Intellectual property / patents
  - Litigation
  - Lack of regulatory harmonization
  - Changes in health economics

Patients Available for Clinical Trials

Example

- **Congenital atransferrinemia**
- **Apotransferrin developed by FRC**
- **2 patients in Finland (population ~ 5M)**
- **Several individuals in USA**
- **FRC's fractionation transferred to Sanquin**
- **Financial viability of technology transfer**
- **Uncertainty about trial requirements**

Clinical Trial Challenges

National organizations

- **Limited numbers of patients (PiD)**
- **Chronic replacement treatment**
- **Willingness to switch ?**
- **Increased visits to hospital**
- **More venepunctures / paperwork**
- **Interruption with life / work**

Clinical Trial Challenges (cont)

National organizations

- **Competition for willing patients**
- **Duration of follow-up**
- **No personal incentive**
- **Comparator ?**
- **Pro-active pharmacovigilance system is easier for small populations**
- **High cost of clinical trials**

Other Considerations

- International commercialization
- Economics of plasma fractionation
- ‘New’ proteins
- Effects on other licensed products
- Collaboration needed between:
  - Patient organizations
  - Physicians
  - Regulators
  - Industry

Final Consideration!

Will health care

purchasers

agree to buy the product for these needy patients ?

Conclusions

- Challenges for small to medium enterprises
- Resources available / investments required
- Patient populations, availability and willingness
- Regulatory harmonization
- International commercialization
- Pro-active pharmacovigilance
- Possible effects on other product licences